Center for Biologics Evaluation and Research

Kathryn C. Zoon, PhD., Director

Forces Shaping Biological Products in the 21st Century

- New Discovery Biomedical Research and Technology
- Demand for New Biological Products and Faster Access
- Safety and Ethical Issues
- Changing Health Care Environment
- Global Market: International Harmonization and Competition; Consolidation/Mergers
- Information Management
- Counter-Terrorism

New Discovery Biomedical Research and Technology

- Genomics/Proteomics
- Cellular Products/Tissue Engineering
- Transgenics/Xenotransplantation
- Gene Therapy


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Human Genome

AND WHAT MIGHT COME OF IT

2005 | 2010 | 2015 | 2020 | 2025

2-03

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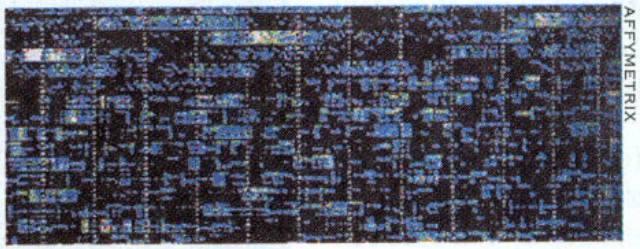
2002-10

- **Tests for risk of CANCER, DIABETES, STROKE**
- **Gene therapy for HEMOPHILIA, HEART DISEASE, some CANCERS**



- **DNA chips that analyze your personal genetic makeup**

2015

- **Medicine tailored to an individual's genetic makeup will be available to treat diseases, including CANCER**

2025

- **Doctors will correct defective genes, making illnesses like SICKLE-CELL ANEMIA a thing of the past**

Source: Dr. Francis Collins (National Human Genome Research Institute)

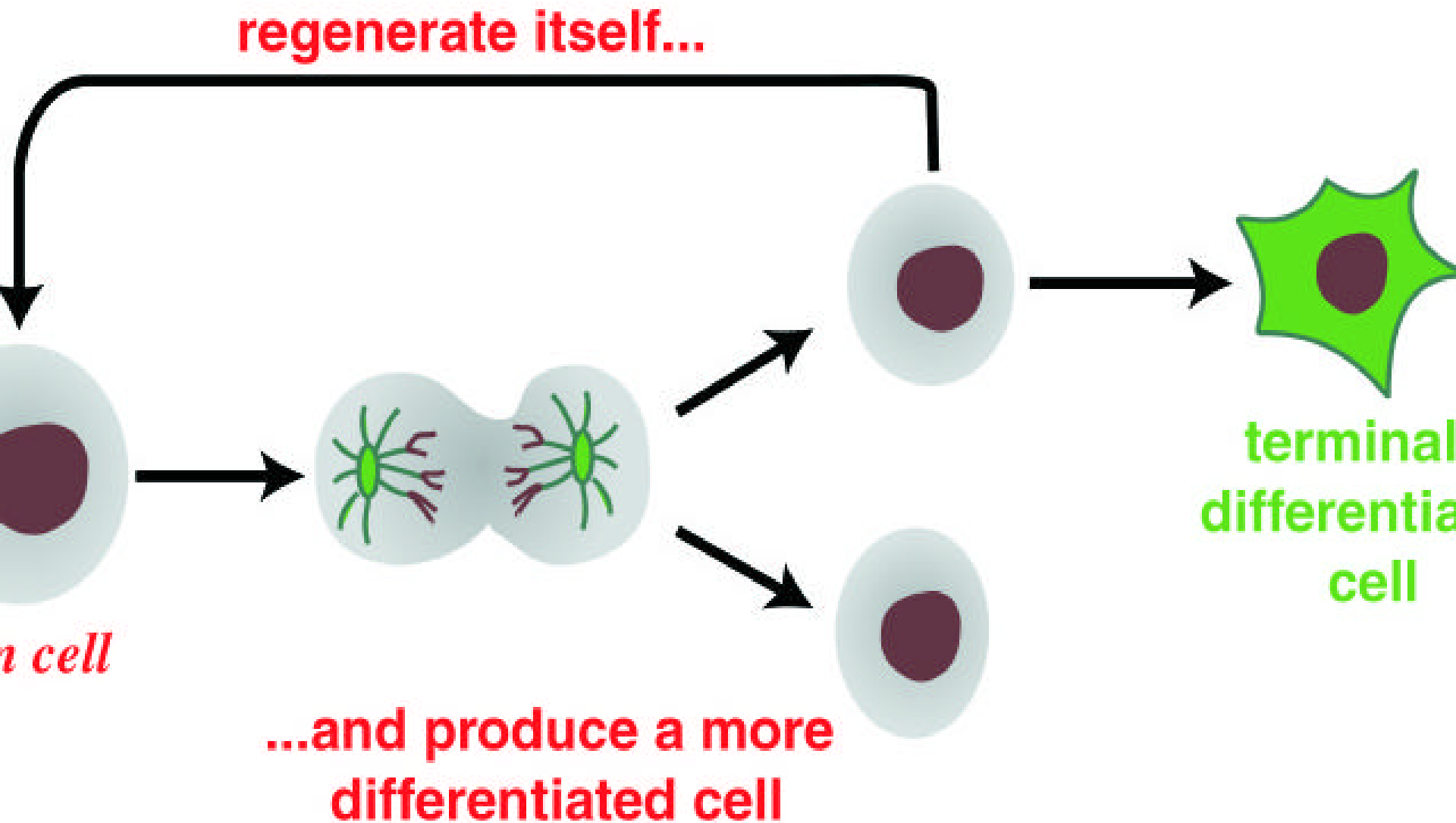
Graphic for TIME by Joe Lertola


ONCE YOU UNFOLD ONE OF THESE THINGS, IT'S NEVER THE SAME.
Joe Heller
Joe@hellertoon.com
MAP OF THE HUMAN GENOME
Ethical Questions
Legal Tangles
Privacy Concerns
Moral Issues
Insurance Applications
Medical DiLemmas

Genomics and Proteomics

- **Impact in the Research Arena:**
  - New Disease Maker-Early Detection
  - New Therapeutic Targets
  - New Markers for Therapeutic Efficacy
  - New Markers for Toxicity
- **Impact in the Regulatory Arena:**
  - New target pool
  - New Markers for Toxicity
  - New Endpoints for Efficacy
  - New Endpoints for Potency
  - Development of New Bioassays, e.g. identity, purity

A ***stem cell*** is one that can



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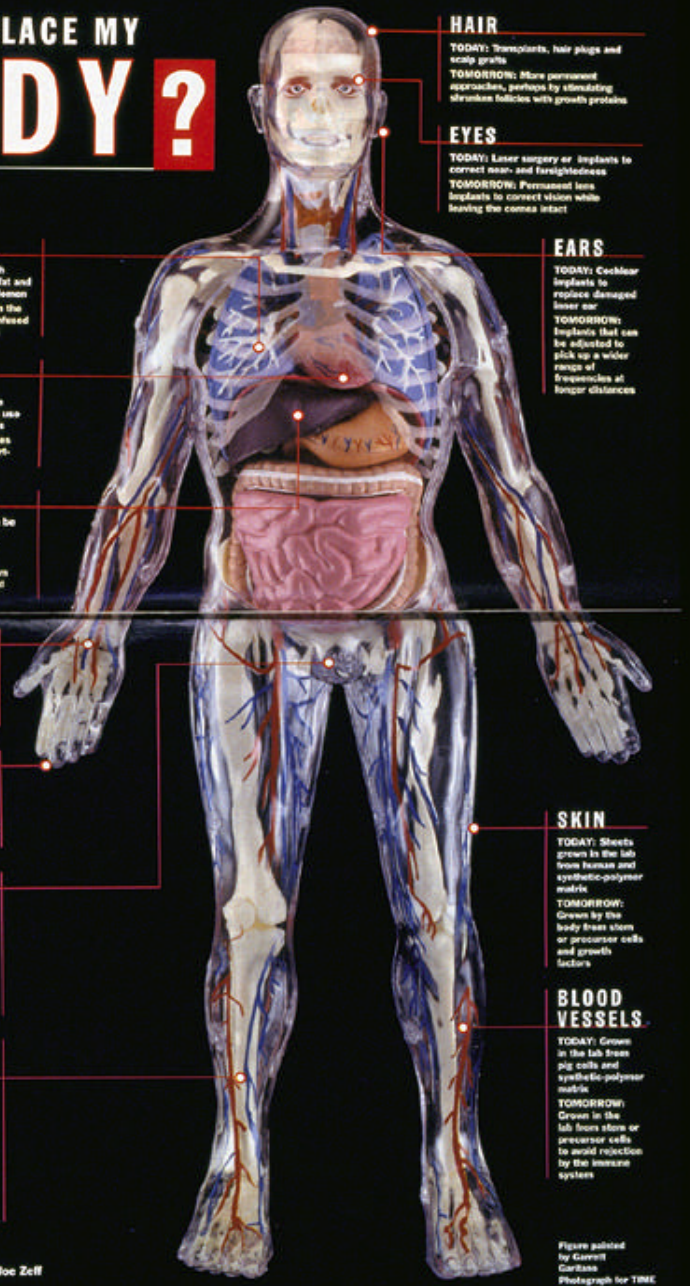
CAN I REPLACE MY BODY?



HAIR

TODAY: Transplants, hair plugs and scalp grafts.

TOMORROW: More permanent approaches, perhaps by stimulating shrunken follicles with growth proteins

EYES

TODAY: Laser surgery or implants to correct near- and farsightedness

TOMORROW: Permanent lens implants to correct vision while leaving the cornea intact

BREAST

TODAY: Breasts are reconstructed with saline sacs or with living tissue, using fat and muscle from the back, buttocks or abdomen

TOMORROW: Breasts may be grown in the lab from a patient's own fat cells and infused back through keyhole slits in the chest

HEART

TODAY: Bypasses, angioplasty and transplants to keep blood flowing to the heart muscle. Doctors are beginning to use gene therapy to grow new blood vessels

TOMORROW: Growing functional patches of heart muscle or coaxing existing heart-muscle cells to repair themselves

ORGANS

TODAY: Small slivers of liver tissue can be grown in the lab from one of the many types of liver cells, but they are not yet ready for transplant

TOMORROW: Heart, liver, kidneys grown from stem cells in vitro and transplanted into the body

EARS

TODAY: Cochlear implants to replace damaged inner ear

TOMORROW: Implants that can be adjusted to pick up a wider range of frequencies at longer distances

NERVES

TODAY: Grown in the lab from pig cells and synthetic-polymer matrix

TOMORROW: Regenerated from stem or precursor cells in the body

LIMBS

TODAY: Prosthetics wired to peripheral nervous system

TOMORROW: Prosthetics wired directly to motor portions of the brain to improve control and simulate the sensations of touch, pain, etc.

PENIS

TODAY: Penile implants and medication to maintain erection. Surgery to reattach a severed penis; skin grafts to recover urinary, but not sexual, function if penis is not recovered

TOMORROW: Genetically engineered tissue grown in the lab and attached for final growth to form fully functional penis

BONE AND CARTILAGE

TODAY: Injection of bone growth factors into jaw and other fracture areas. Researchers can also grow cartilage in the lab in thin sheets, but it's too weak to be functional in the body

TOMORROW: Coaxing the body to grow bone and cartilage on biodegradable scaffolds infused with a mix of stem cells and growth factors

SKIN

TODAY: Sheets grown in the lab from human and synthetic-polymer matrix

TOMORROW: Grown by the body from stem or precursor cells and growth factors

BLOOD VESSELS

TODAY: Grown in the lab from pig cells and synthetic-polymer matrix

TOMORROW: Grown in the lab from stem or precursor cells to avoid rejection by the immune system

By Alice Park; TIME Graphic by Joe Zeff

Figure painted by Garrett Caritas
Photograph for TIME by Ted Thai

Vaccines of the 21st Century

- **New Vaccines**
  - **DNA Vaccines**
  - **Tumor Vaccines**
  - **Live Attenuated Vaccines**

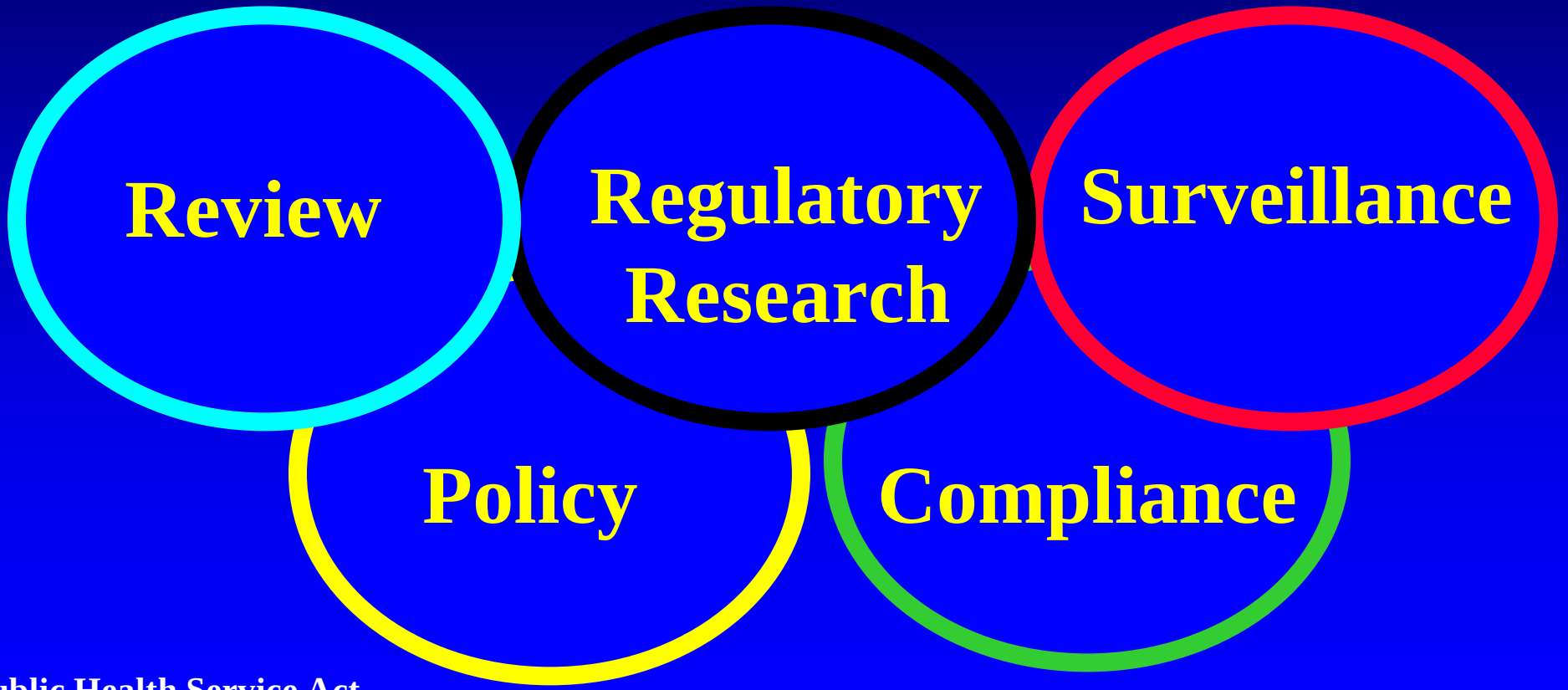
Blood Safety in the 21st Century

- New Blood Screening Tests
- Pathogen Inactivation
- Blood Substitutes

COUNTER- BIOTERRORISM

Current and Future Regulation of Biological Products

Based on Sound Science, Law and Public Health Impact



ublic Health Service Act

Food Drug and Cosmetic

Destiny is not a matter of chance; it is a matter of choice. It is not something to be waited for; but rather something to be achieved

William Jennings Bryan